

Lupus Patient-Focused Drug Development (PFDD) Initiative

Presented to the NIAMS Coalition

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Patient-Focused Drug Development (PFDD) Initiative

- People living with diseases are uniquely positioned to inform understanding of the therapeutic context for drug development and evaluation.
- There is a need for more systematic ways of gathering patient perspective on their condition and treatment options.
- Current mechanisms for FDA to obtain patient input often limited to discussions related to specific applications under review.

Criteria for FDA Public Meeting

- Disease areas that are chronic, symptomatic, or affect functioning and activities of daily living.
- Disease areas for which aspects of the disease are not formally captured in clinical trials.
- Disease areas for which there are currently no therapies or very few therapies, or the available therapies do not directly affect how a person feels, functions, or survives.
- Disease areas that have a severe impact on identifiable subpopulations (such as children or the elderly).

People With Lupus Need New Treatments

Significant unmet need and current standards of care are unacceptable:

- Only four treatments currently approved for lupus
- Most medications used are drugs approved for other indications—chemotherapies for cancer, biologics for forms of arthritis, and immunosuppressants used post organ transplantation
- Many drugs cause significant side effects: infections, cancer, bone loss, osteoporosis, sterility, stroke, diabetes, glaucoma, birth defects, and infertility.

Our PFDD Journey - Part 1

- October 2014 Federal Registrar Notice requesting input for additional diseases to be the focus of future FDA Public Meetings
- Each organization responded to the Notice
- Co-led a lupus community call to action requesting comments from people with lupus, family members, advocates, clinicians, and researchers
- Many comments on lupus were submitted by the December 2014 deadline

Our PFDD Journey - Part 2

- Lupus was **not chosen** as a disease area to be the focus of a future FDA Public Meeting.
- The three organizations decided to collaborate and submitted a Letter of Intent (LOI) in November 2015 to lead an externally-led Lupus PFDD Initiative.
- The FDA responded favorably and supports us in this endeavor.
- We are responsible for executing the entire initiative.

Lupus PFDD Key Dates

- May 23, 2017 - Website, Online Survey, and Meeting Registration Launch LupusPFDD.org
- September 25, 2017 – *Lupus: Patient Voices* Meeting at College Park Marriott Hotel and Conference Center in Hyattsville, MD 10 am-4 pm
- Early 2018 - *Lupus: Patient Voices Report* Release

Lupus PFDD Action Plan

- Timeline
- Memo of Understanding (MOU)
 - ✓ Project Treasurer
 - ✓ Points of Contact for FDA, Industry Sponsors, Researchers
- Communications Document
- Sponsor Packets
- Research Funding Proposal (RFP) for Project Manager
 - ✓ Website Design
 - ✓ Survey Development and Analysis
 - ✓ Panelist and Audience Participant Selection and Preparation
 - ✓ Executing Meeting (moderator, venue, travel, A/V, webcast, polling)
 - ✓ Developing *Lupus: Patient Voices Report*
- Plan B

Lupus PFDD Initiative Captures Patient Viewpoints

Patient input on:

- Burden of disease; daily impacts on quality of life
- Patient assessments of current and potential future treatments
- Benefits that people with lupus are looking for in terms of treatments and the risks that they are willing to assume in return for more advanced interventions
- Views on participation in clinical trials and ideas on trial design to make studies more appealing to people with lupus
- Information incorporated into a benefit-risk assessment that will be used in the drug review process

Why This is Important

Clinical trials are not designed to measure those outcomes which have been described as most important to those living with lupus such as:

- mitigating fatigue
- aspects of daily health related quality of life
- complex drug regimen
- corticosteroid sparing
- intolerable side effects
- emerging comorbid conditions

Significance for People with Lupus

Many people with lupus are concerned with potential side effects of drugs such as hair loss, skin rashes, weight gain, and gastrointestinal problems as well as disease progression; outcomes that may be emotionally and socially challenging to young people in the prime of their lives.

Goals of the Lupus PFDD Initiative

1. To understand the lupus patient journey and recognize patient preferences and risk tolerance.
2. To examine the complexity and heterogeneity of the disease to help better inform trial designs.
3. To create a practical, scientifically rigorous framework that incorporates patient preferences and patient reported outcomes into lupus clinical research and trials.
4. To ensure people with lupus understand the value of their participation in the drug development and clinical trials process by acknowledging how their contributions impact decision-making and outcomes.

Pre-Meeting Survey

The pre-meeting survey:

- was available online and in paper form
- was available in Spanish
- gathered information from nearly 2,200 people with lupus and representatives of children with lupus
- assisted in the planning of the September meeting content and in the selection of participants
- will contribute to the development of the report to the FDA post-meeting

Diverse Patient Population

The survey respondents, meeting panelists and audience participants were inclusive of people with lupus and parents of children with lupus representing individuals who:

- Spanned the spectrum of lupus, such as: systemic lupus erythematosus (SLE); discoid lupus erythematosus; subacute cutaneous lupus erythematosus; and lupus nephritis
- Ranged from the recently diagnosed to those who had lived with lupus for many years
- Experienced symptoms for years but were undiagnosed
- Represented the various subsets of race and ethnicities significantly and adversely impacted by lupus

Diverse Patient Population (cont'd)

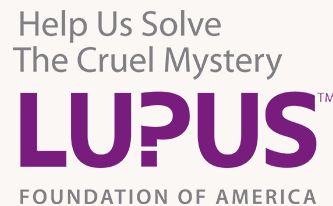
- Had symptoms ranging from mild to severe
- Experienced organ system involvement such as: joints, skin, kidneys, heart, lungs, blood vessels, brain, blood, GI tract, nerves
- Received various treatments and impacts of these current treatments on their bodies and quality of life
- Had well-controlled symptoms on such treatments and those whose symptoms were poorly controlled.

This ensured a robust and diverse patient experience was heard by the FDA and other stakeholders.

LUPUS PATIENT-FOCUSED DRUG DEVELOPMENT MEETING

SEPTEMBER 25, 2017

Presented By



Pre-Meeting Dinner

An opportunity for the three convening organizations, patient participants and their representatives, PFDD Team members, and the sponsors to gather in celebration of the lupus community's historical endeavor on the evening before the meeting.



Lupus: Patient Voices Meeting

Day-long meeting featured:

- Two moderated six-person patient panels
- Two facilitated patient audience discussions
- Live polling from patient participants via audience and webinar
- Open public comment session
- Over 550 in-person and webinar attendees.



Meeting Agenda

- **Welcome and Opening Remarks**
- **Background**
 - Overview of FDA's PFDD Initiative and Clinical Trial Endpoints
 - Interview with Janet Woodcock, M.D., Director, FDA Center for Drug Evaluation and Research
 - Overview of Lupus and Available Treatments
 - Overview of Discussion Format
- **Discussion Topic 1:** Patient perspectives on disease symptoms and daily impacts that matter most
- **Lunch**
- **Discussion Topic 2:** Patient perspectives on current approaches to treating lupus
- **Open Public Comment Period**
- **FDA Summary Remarks**
- **Closing Remarks**

Topic 1 Discussion

Patient perspectives on disease symptoms and daily impacts that matter most

- Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
- Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition?
- How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days?
- How have your condition and its symptoms changed over time?
- What worries you most about your condition?

Topic 2 Discussion

Patient perspectives on current approaches to treating lupus

- What are you currently doing to help treat your condition or its symptoms?
- How well does your current treatment regimen control your condition?
- What are the most significant downsides to your current treatments, and how do they affect your daily life?
- Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?
- What factors do you take into account when making decisions about selecting a course of treatment?

Lupus: Patient Voices Report

A final written report of the meeting and its findings will be distributed to the FDA, interested stakeholders, and the general public. The report will:

- Offer an overview of the meeting, combining key themes and capturing information from any surveys conducted
- Capture the nuances and subtleties of the patient discussions and the complexity of lupus
- Provide a well-rounded, fully developed understanding of patient preferences and risk tolerance.

Our Promotion

- Share the launch announcement with your lupus networks.
- Promote taking the survey for people with lupus.
- Encourage participation in the live meeting in person or virtually via webcast.
- Register to attend in person or via webcast.
- Promote the survey and meeting at every possible opportunity.

Helpful Hints

- Have a communications plan that includes announcements promoted through e-blasts and social media links and sample posts with a timeline
- Use social media to drive people to the website to take the survey and register for the meeting and webinar
- Think about any sub-groups in your population that are traditionally hard to reach and have a plan to target them for participation
- Have diversity but expertise among the Steering Committee Members
- Work closely with the FDA to schedule the date and invite speakers with enough time for them get appropriate approvals

Helpful Hints (cont'd)

- Use survey results to help drive meeting content including live polling questions
- Speak with other organization leaders who held PFDD Meetings
- Attend PFDD Meetings in-person or via webcast and if you can't, then watch the videos of previously held PFDD Meetings
 - Eg. SMA - which was held at the same venue and used the same A/V company we did
- Prepare the panelists – have them draft their remarks and practice
 - Steering committee and moderator should provide constructive feedback, other panelists can provide input as well

Visit LupusPFDD.org

- Watch the meeting recording, learn about the survey, see the meeting announcements, and more

The screenshot displays the LupusPFDD.org website. At the top, the navigation bar includes the LupusPFDDMeeting logo, the date and location (September 25, 2017, College Park Marriott / Hyattsville, MD), a LIVE WEBCAST button, and links for Home, About, Meeting, Organizers, En Español, and Contact Us. The main content area features a large video player for the 'Lupus Patient-Focused Drug Development Meeting Webcast'. The video title is 'LUPUS PATIENT-FOCUSED DRUG DEVELOPMENT MEETING' with the date 'SEPTEMBER 25, 2017'. Below the video, the 'Presented By' section lists three organizations: LUPUS ALLIED DISEASES Association, Inc., LUPUS FOUNDATION OF AMERICA (with the tagline 'Help Us Solve The Cruel Mystery'), and LUPUS RESEARCH ALLIANCE. At the bottom, there is a section for 'Downloadable Resources' with a link to 'Lupus PFDD Meeting Agenda.pdf'. The footer contains the text 'GET INVOLVED' with a right arrow and social media icons for YouTube, Instagram, and Facebook, along with the hashtag #LUPUSPFDD.

LupusPFDDMeeting

September 25, 2017
College Park Marriott / Hyattsville, MD

LIVE WEBCAST

Home About Meeting Organizers En Español Contact Us

Lupus Patient-Focused Drug Development Meeting Webcast

LupusPFDDMeeting

**LUPUS PATIENT-FOCUSED
DRUG DEVELOPMENT MEETING**

SEPTEMBER 25, 2017

Presented By

LUPUS ALLIED DISEASES Association, Inc.

Help Us Solve
The Cruel Mystery
LUPUS
FOUNDATION OF AMERICA

LUPUS RESEARCH ALLIANCE

Downloadable Resources
[Lupus PFDD Meeting Agenda.pdf](#)

GET INVOLVED >

YouTube Instagram Facebook #LUPUSPFDD

Thank You

The contribution of the patient perspective is one that stimulates and challenges but also enriches and instructs the process all along the regulatory and development pathways.

